

GLOBAL BURDEN OF WALDENSTRÄM MACROGLOBULINEMIA (WM): A SYSTEMATIC LITERATURE REVIEW (SLR) AND EVIDENCE GAP ANALYSIS

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OBJECTIVES: To review the global clinical, economic, and humanistic burden of WM and evaluate current evidence data gaps.

METHODS: A SLR using PubMed to identify WM-related interventional, observational, qualitative, and economic studies published January 2013-September 2018. Inclusion criteria: human clinical studies, N>40, non-case reports or editorials, English manuscripts available.

RESULTS: Of 1146 evaluable publications, 51 were included based on above search criteria. WM incidence ranged from 0.3-0.57/100,000 person-years in US studies, with most data showing decreased or stable incidence over time. WM incidence appeared similar in Japan and Taiwan but higher in Sweden (1.05/100,000). Recent data trends suggest significantly increased WM-related survival, though 5-year survival rates vary (52%-78%) in US studies. European data demonstrate similar survival rates and trends (5-year survival ~70%) but lower survival in China (61.8%). Across countries, WM appears to be more common in men and in elderly. Mortality rates are also shown to be higher in elderly and male patients. US data suggest higher incidences in Caucasian patients but reduced survival for African American and Hispanic patients. Compared to Westerners, Chinese patients demonstrated a higher male-to-female ratio, younger diagnosis age, higher rates of leukocytopenia, thrombocytopenia, and pancytopenia, and reduced asymptomatic WM. WM-related serious complications are reported, including secondary malignancies, thrombosis, renal diseases, symptomatic hyperviscosity, and autoimmune manifestations. Although novel treatments contribute to improved survival, they significantly increase costs (mean Medicare costs in first treatment year: \$9,464 before 2000 to \$29,490 after 2008; most recent data available). Data on the humanistic burden of WM are lacking.

CONCLUSIONS: This study reviewed the burden of WM across the regions (US, EU, and China). Although rare, WM results in significant clinical (e.g., incidence, complications, survival) and economic burden and unmet needs, with new treatments associated with clinical benefits but increased costs. Data gaps on WM-burden exist, future studies are suggested.